



Advancing Clinically Validated Platform Toward Commercialization

44TH ANNUAL J.P. MORGAN HEALTHCARE CONFERENCE | JANUARY 2026



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Poised to Unlock Significant Commercial Opportunities in Multiple Rare Neuromuscular Diseases



LATE-STAGE CLINICAL PIPELINE

- Positive topline results from registrational cohort in DMD
- Ongoing registrational cohort in DM1



NEAR-TERM VALUE DRIVERS

Steady cadence of expected data readouts and regulatory submissions; first potential commercial launch in Q1 2027



DIFFERENTIATED PLATFORM

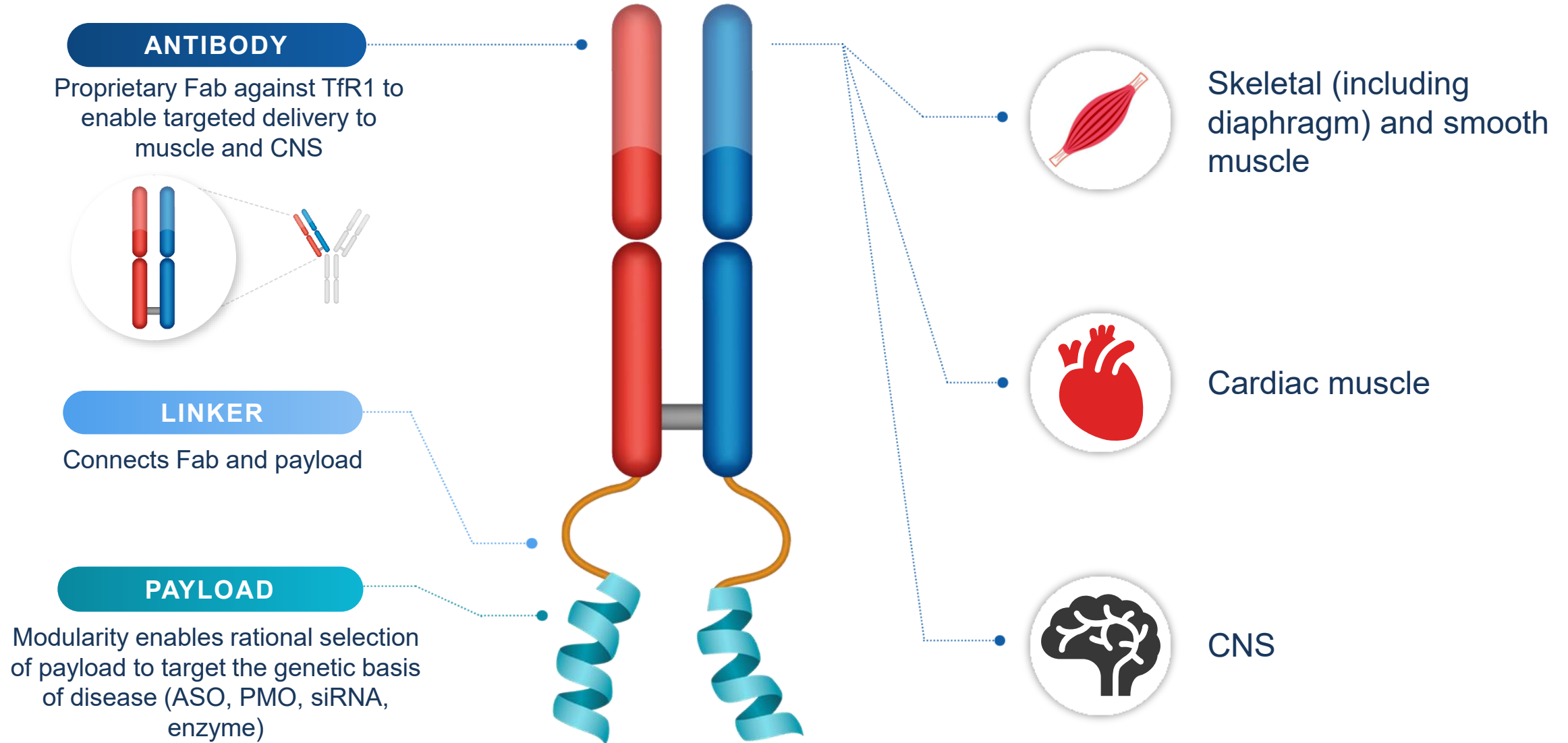
FORCE™ platform enables targeted delivery to muscle and CNS; broader pipeline includes FSHD and Pompe



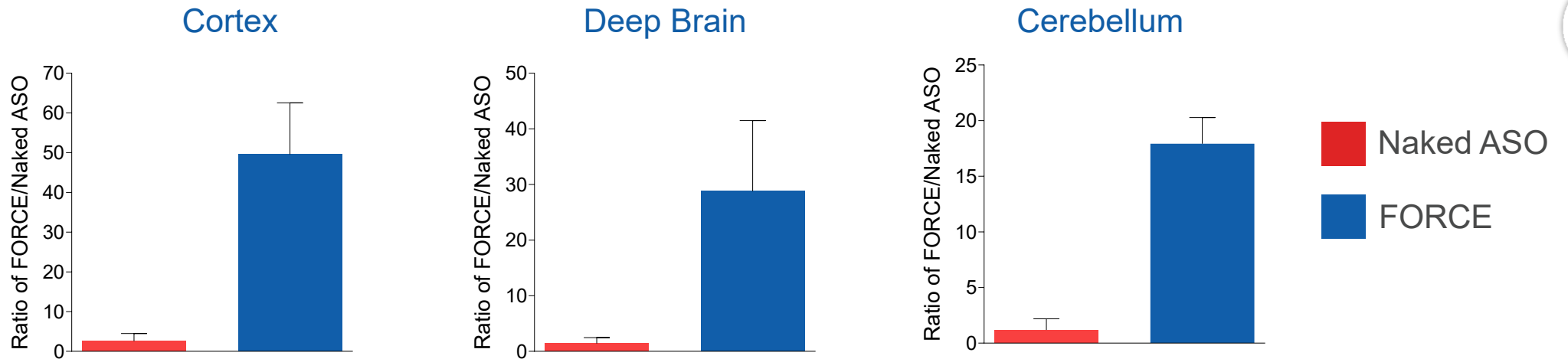
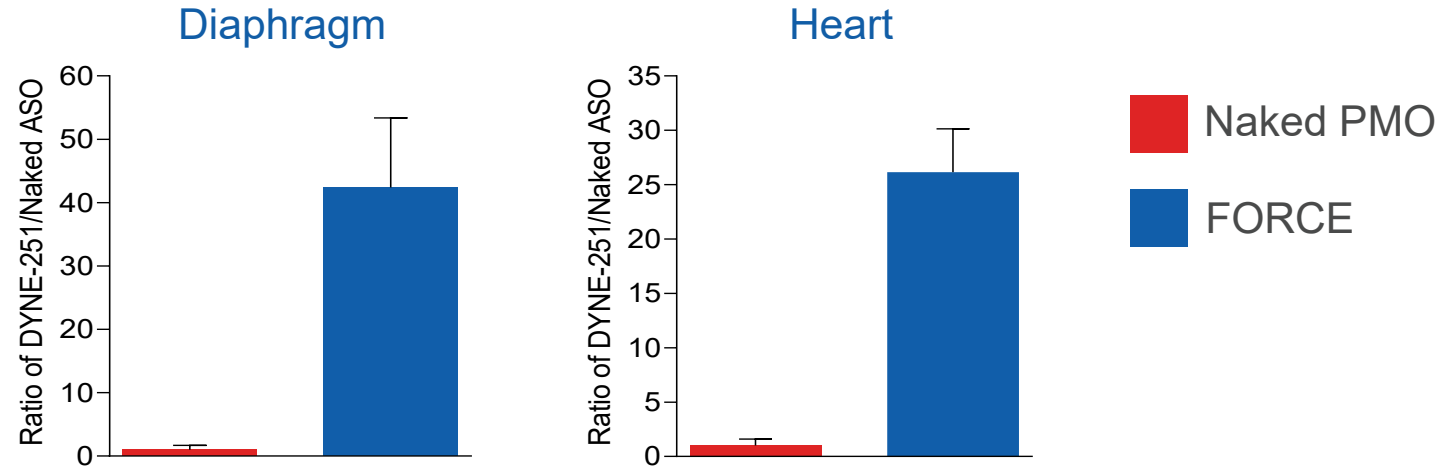
STRONG FINANCIAL POSITION

Cash position of ~\$1.1 billion (as of 12/31/25)¹ with expected runway into Q1 2028; all assets fully owned

Leveraging Our FORCE™ Platform for Targeted Delivery

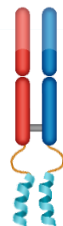


FORCE Enabled Oligonucleotide Delivery to Muscle and CNS



Potential of the FORCE Platform Validated by Recent Z-Rostudirsen Topline Clinical Results

FORCE



Design Principles of the FORCE Platform

TfR1-mediated delivery to muscle, including diaphragm and heart, and CNS with rationally selected payload to match disease biology

TfR1-binding Fab to enable robust and widespread tissue distribution

Designed not to interfere with TfR1 function in iron homeostasis

Achievement of target profile with infrequent dosing

Validation with Z-Rostudirsen DELIVER Data

Statistically significant and robust increase in dystrophin

Early and sustained functional improvement across multiple clinical endpoints

Favorable safety and tolerability¹ with no persistent related anemia² or thrombocytopenia at 20 mg/kg

Convenient Q4W dosing

Dyne's pipeline programs utilize the same TfR1-binding Fab

Neuromuscular Pipeline Leveraging Clinically Validated Platform

DISEASE	TARGET	PRECLINICAL	PHASE 1/2	ESTIMATED PATIENTS
Myotonic dystrophy type 1 (DM1)	DMPK	zeleciment basivarsen (z-basivarsen, also known as DYNE-101)		US: ~40,000 EU: ~55,000
Duchenne muscular dystrophy (DMD)	Exon 51	zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251)		US: ~12,000 EU: ~16,000
	Exon 53			
	Exon 45			
	Exon 44			
	Other Exons			
Facioscapulohumeral muscular dystrophy (FSHD)	DUX4	DYNE-302		US: ~15,000 – 40,000 EU: ~20,000 – 50,000
Pompe disease	GAA	DYNE-401		US: ~4,500 EU: ~5,500

PIPELINE EXPANSION OPPORTUNITIES

CNS, Rare skeletal, Cardiac, Metabolic



Broad and Durable Functional Improvement Observed with Dyne's First Clinically Validated Program: Z-Rostudirsen in DMD



Exon 51 Skip Amenable DMD: A More Severe Duchenne Population with Significant Unmet Need, Despite Approved Therapies



DMD Population

- ~12,000 (US)
- ~16,000 (EU)
- ~ 13% is exon 51 skip amenable¹



Clinical Presentation

- Mutation in *DMD* gene for dystrophin
 - Exon 51 skip amenable DMD is a particularly challenging form
- Muscle weakness and gait abnormalities
- Progressive loss of function
- Cognitive issues
- Respiratory/cardiac failure
- Life expectancy ~30 years²



Current Treatment Limitations

- Limited delivery to muscle and CNS
- High burden due to weekly IV dosing³
- <1% dystrophin production with exon 51 skipping therapy³
- Microdystrophin lacks domains key for optimal functionality⁴
- Unknown durability and inability to redose with gene therapy
- Safety considerations



OUR APPROACH

Potential Best-in-class Targeted Exon Skipping

Increase dystrophin expression and enable less frequent dosing to deliver **functional improvement**

DELIVER Study to Support Accelerated Approval of Z-Rostudirsen in DMD



Selection of registrational dose (20 mg/kg Q4W) based on multiple ascending dose (MAD) data



Registrational Expansion Cohort met primary endpoint of statistically significant and robust increase in dystrophin at 6 months ($p < 0.0001$)



Functional improvement observed across multiple clinical measures out to 24 months



Favorable safety profile¹

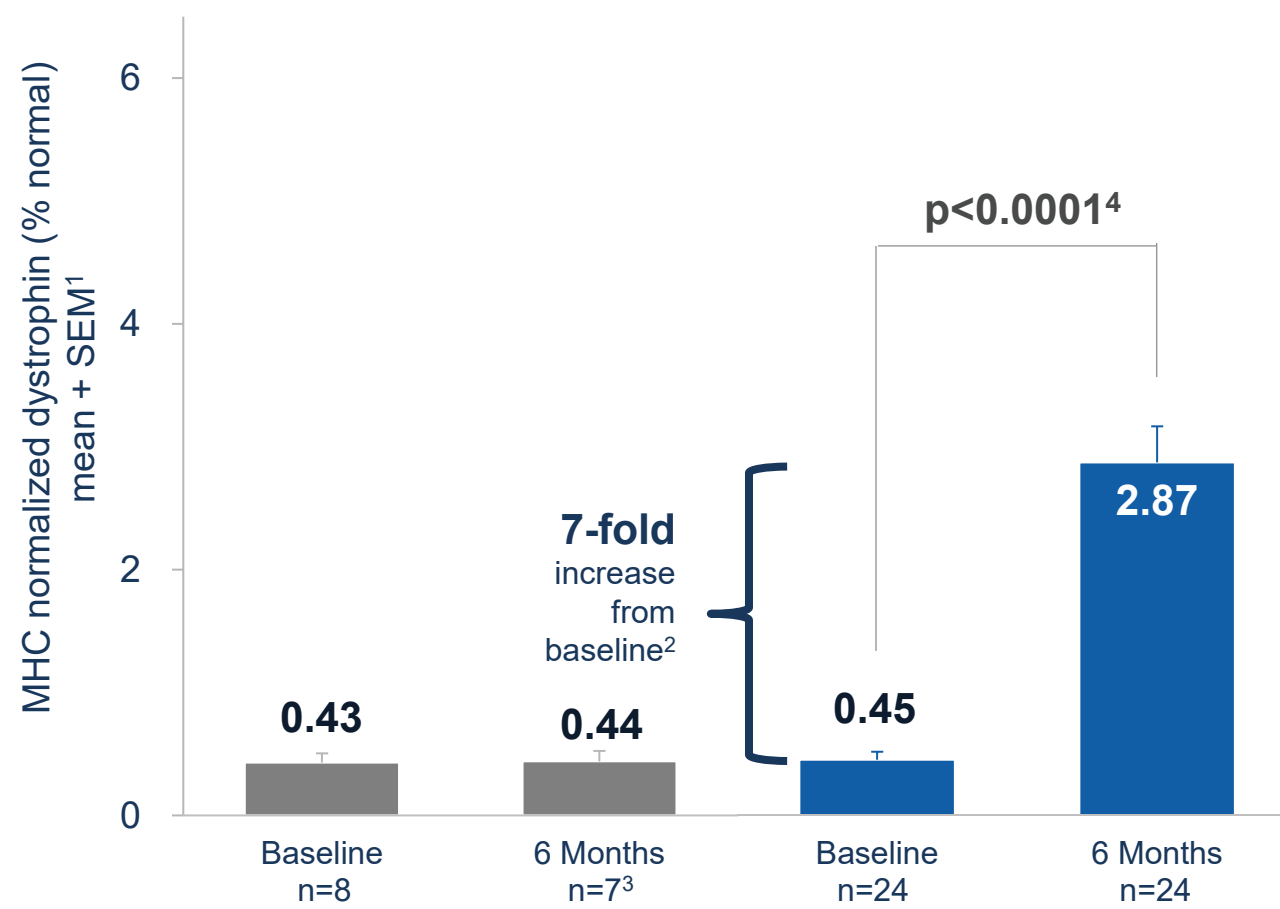


Planned submission for U.S. Accelerated Approval based on positive results from Registrational Expansion Cohort

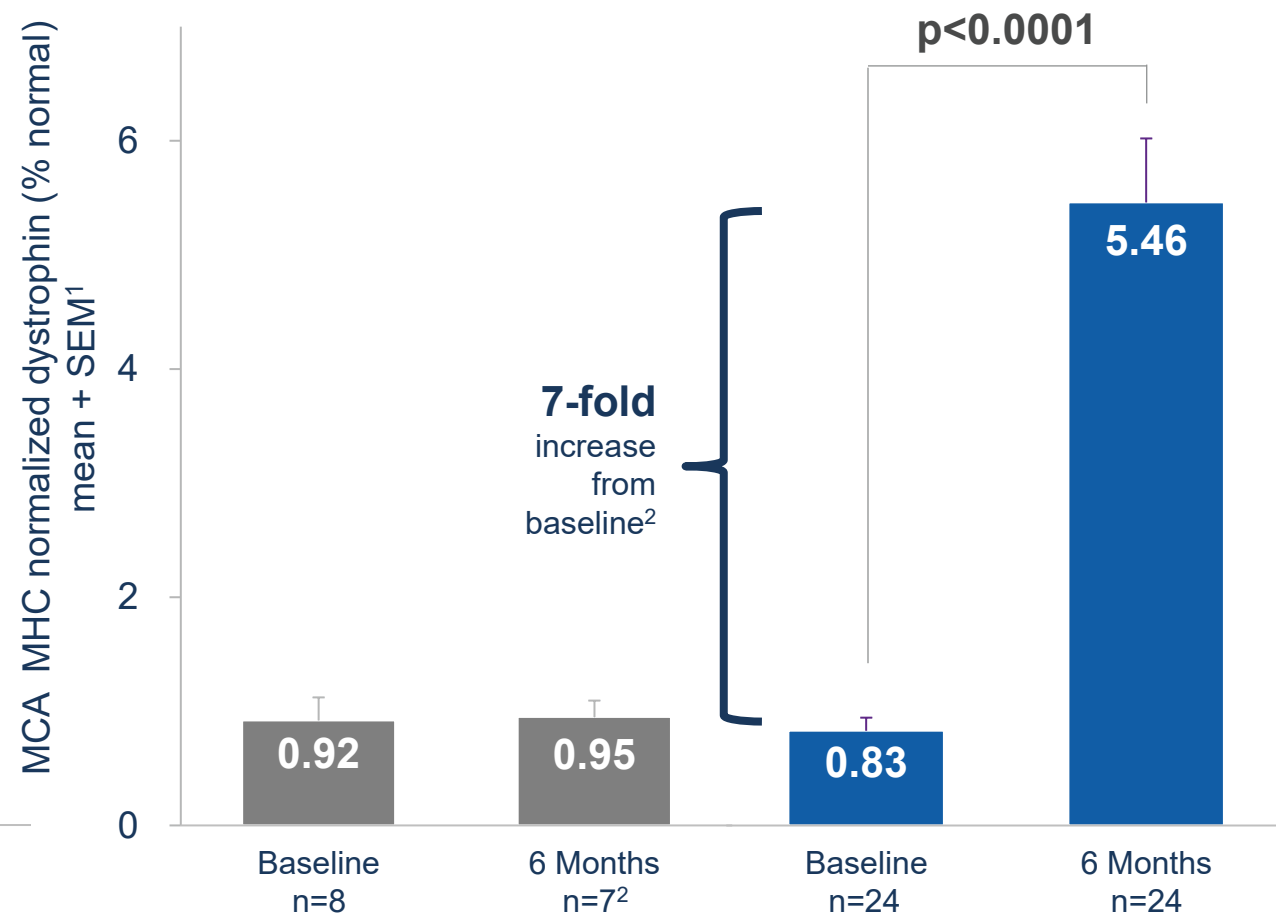
Phase 3 study planned to support full approval of z-rostudirsen globally

Z-Rostudirsen Achieved a Statistically Significant and Robust Increase in Dystrophin Expression at 6M in Registrational Expansion Cohort

Unadjusted dystrophin



Muscle content-adjusted dystrophin⁵

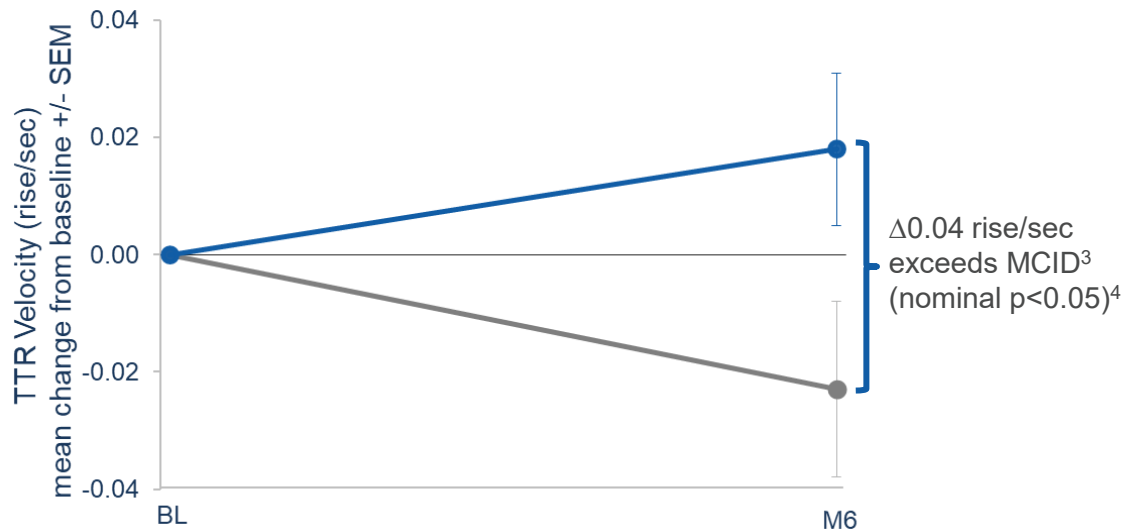


■ Placebo

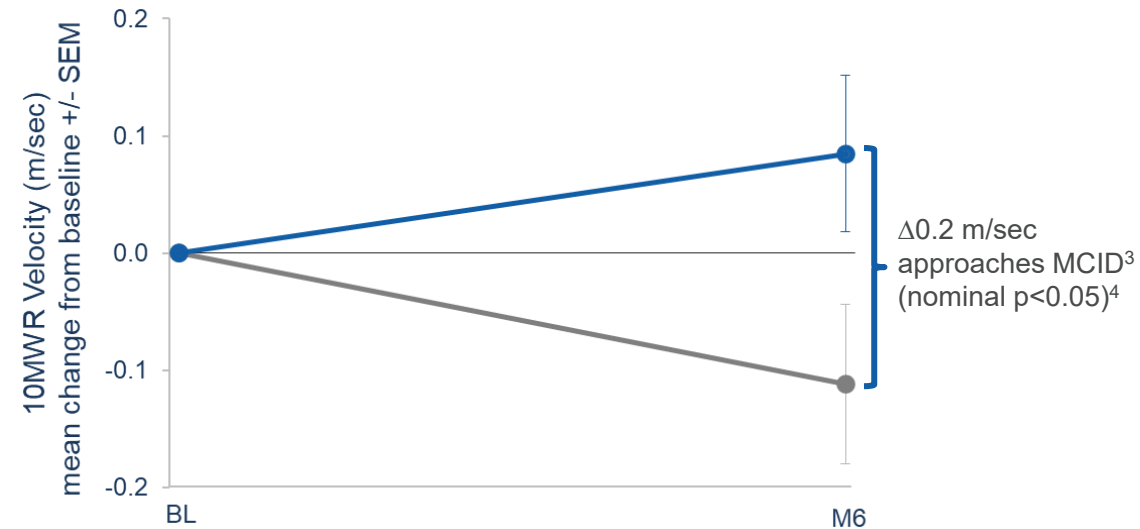
■ Z-rostudirsen 20 mg/kg Q4W (REC)

Improvement in TTR Velocity and 10MWR Velocity at 6 Months Relative to Baseline and Placebo

Time to Rise¹ (TTR) Velocity²



10-Meter Walk/Run (10MWR) Velocity²

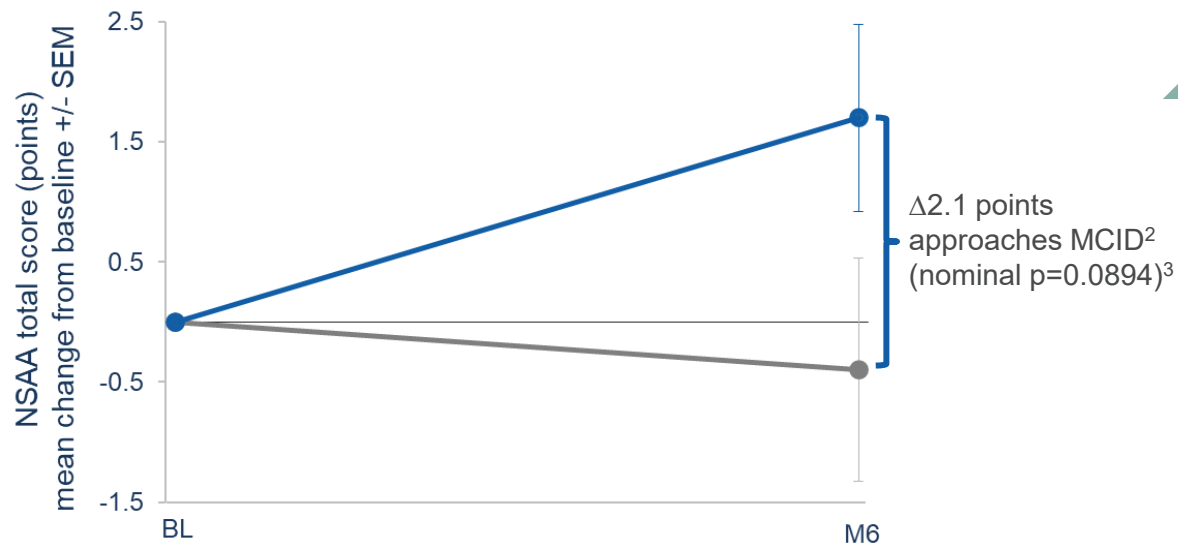


- Placebo (REC+MAD) (n=18)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=21)

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- Z-rostudirsen 20 mg/kg Q4W (REC) (n=21)

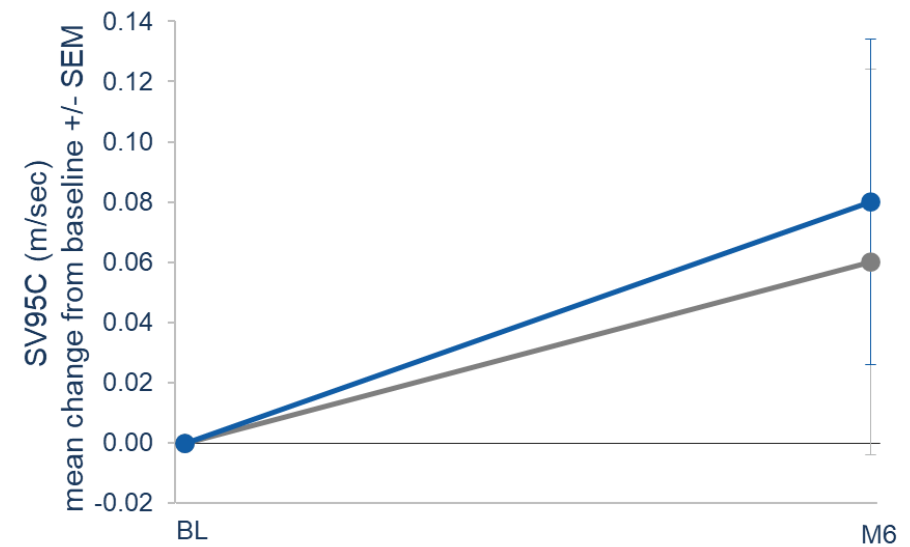
Improvement in NSAA and SV95C at 6 Months Relative to Baseline

North Star Ambulatory Assessment (NSAA)¹

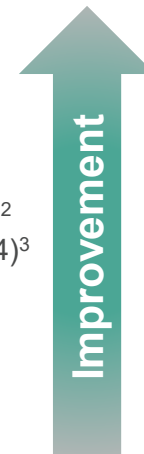


- Placebo (REC+MAD) (n=18)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=21)

Stride Velocity 95th Centile (SV95C)¹

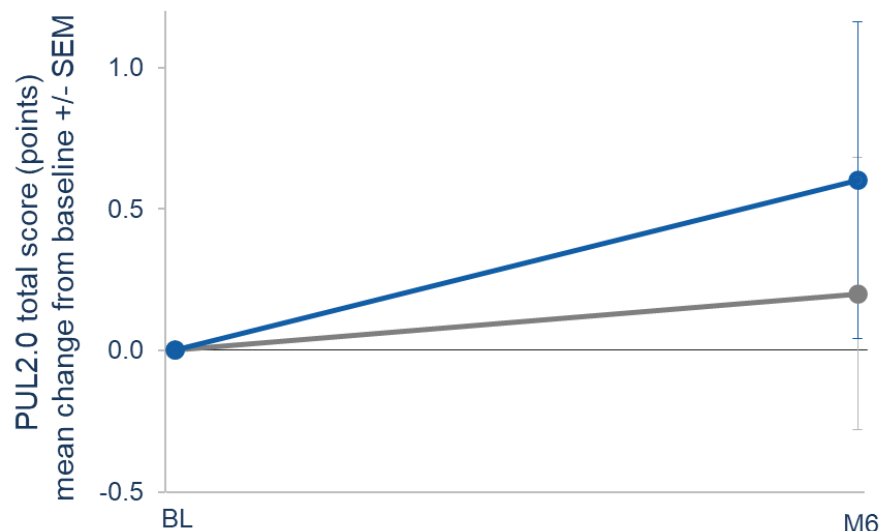


- Placebo (REC+MAD)⁴ (n=12)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=20)

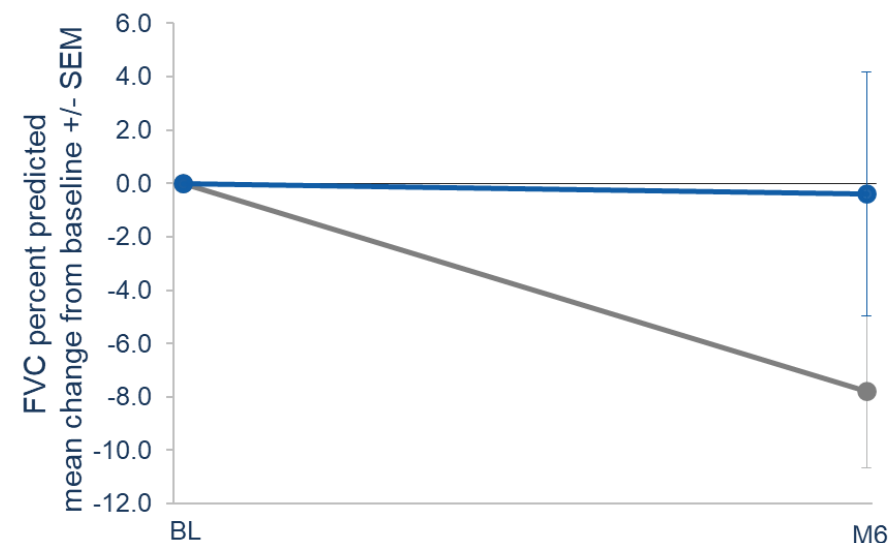


Improvement in PUL2.0 at 6 Months Relative to Baseline and Placebo; Preservation of Lung Function at 6 Months

Performance Upper Limb v2.0 (PUL2.0)¹



Forced Vital Capacity Percent Predicted (FVC%p)¹



- Placebo (REC+MAD) (n=23)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=22)

- Placebo (REC+MAD) (n=20)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=15)

Unprecedented Breadth and Durability of Functional Improvement with Z-Rostudirsen

BREADTH			DURABILITY	
Endpoint	Patient Population	Muscle System	6-month Functional Improvement vs. Placebo ¹	24-month Functional Improvement vs. Baseline ²
TTR Velocity	Ambulatory	Trunk & lower limbs	✓	✓
10MWR Velocity	Ambulatory	Lower limbs	✓	✓
NSAA	Ambulatory	Upper limbs, trunk & lower limbs	✓	✓
SV95C	Ambulatory	Lower limbs	✓	✓
PUL2.0	Ambulatory & non-ambulatory	Upper limbs	✓	✓
FVC%p	Ambulatory & non-ambulatory	Diaphragm & trunk	✓	✓

Z-Rostudirsen: Favorable Safety Profile

Summary of treatment-emergent adverse events (TEAEs)¹

Study Period	Placebo-Controlled (PC) Period (0 to 6M)		All Study Periods (0 to ≤36M)
	Placebo (MAD+REC) N=24 ²	Z-rostudirsen 20 mg/kg Q4W (MAD+REC) N=30 ³	
Participants with ≥1 TEAE – n (%)			Z-rostudirsen Pooled doses ⁴ (MAD+REC) N=85 ⁵
Any TEAE	22 (91.7)	29 (96.7)	80 (94.1)
Any related TEAE	3 (12.5)	10 (33.3)	41 (48.2)
Any serious TEAE	1 (4.2)	2 (6.7)	10 (11.8)
Any serious related TEAE	0	0	4 (4.7)
Any TEAE leading to withdrawal from study	0	0	0
Any TEAE leading to death	0	0	0

Most related TEAEs were mild or moderate

Potentially related serious TEAEs

- 2 participants at 20 mg/kg Q4W (registrational dose)
 - Pyrexia (fever) and malaise⁶
- 2 participants at 40 mg/kg Q4W
 - Acute kidney injury; thrombocytopenia⁷
 - Pancytopenia⁸

Most frequent related TEAEs ≥10%⁹

- Pyrexia (fever) (18%)
- Headache (13%)

Additional safety data at 20 mg/kg Q4W

- No participants have persistent related anemia¹⁰ or thrombocytopenia

1,441 doses of z-rostudirsen administered to date representing 113 patient-years of follow-up (up to 36 months)¹
1,062 doses of z-rostudirsen at 20 mg/kg dose level administered to date¹

1. Data as of August 19, 2025; all participants, placebo-controlled period, OLE, and LTE. 2. All placebo participants pooled from MAD and REC. 3. All participants randomized to z-rostudirsen 20 mg/kg Q4W in MAD and REC cohorts. 4. All doses of z-rostudirsen from MAD and REC at doses ranging from 0.7 mg/kg to 40 mg/kg every 4 or 8 weeks. 5. One participant randomized to placebo in REC not yet dosed with z-rostudirsen as of August 19, 2025. 6. One participant with same day onset of pyrexia and malaise in OLE and separate single event of pyrexia in LTE; one participant with single event of pyrexia in LTE; both participants fully recovered and have continued to receive z-rostudirsen without interruption. 7. Events had same day of onset in a single participant with a non-serious related TEAE of anemia in the context of fever, hemolysis, diarrhea, and positive blood in stool; together these events were consistent with hemolytic uremic syndrome with a possible infectious etiology. 8. Participant has a history of hemolytic anemia of unidentified etiology; presented with fever and tonsillitis; symptoms resolved without therapeutic intervention. 9. All cohorts combined; preferred terms reported. 10. No participants have persistent related anemia with Hgb levels <11.2 g/dL (threshold for anemia in children (ref: Powers JM. Approach to the child with anemia. UpToDate, Connor RF (Ed), Wolters Kluwer. Accessed December 2, 2025)). M, months; MAD, multiple ascending dose; REC, registrational expansion cohort; Q4W, every 4 weeks; OLE, open-label extension; LTE, long-term extension.

Z-Rostudirsen Offers a Compelling Profile for Potential Accelerated Approval and Addressing Unmet Need in DMD

Z-Rostudirsen: Potential Best-in-class Profile for Exon 51 DMD



Statistically Significant and Robust Increase in Dystrophin at 6 Months



Favorable Safety & Tolerability Profile up to 36 Months¹



Functional Improvement Observed Across Multiple Clinical Measures



Convenient Q4W Dosing

“I am highly encouraged by these new results from the placebo-controlled Registrational Expansion Cohort and the longer-term portions of DELIVER, and I look forward to being able to offer z-rostudirsen to eligible DMD patients, if approved.” - Perry Shieh, M.D, Ph.D.

Preparing to Launch into an Established Rare Disease Market with Well-Characterized Patient Population and Treatment Centers

Well-Characterized Patient Population

>50%

Exon 51 skip amenable patients have been treated with a disease modifying therapy¹

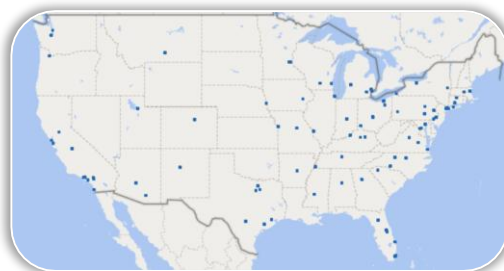
- ~12,000 US DMD population
 - ~1,600 US Exon 51 skip amenable DMD
- Active and educated patient community
- HHS added DMD to RUSP in December 2025

Concentrated Treatment Centers¹

~80%

of DMD patients cared for at top 100 DMD centers

- Over 80% of top 100 centers have experience prescribing DMTs to exon 51 patients



- Significant overlap expected with DM1 centers

Established Market with Reimbursement

~\$1M

WAC price of currently approved exon skippers for average patient per year²

- First exon skipper approved in 2016
- Established reimbursement pathways and clear recognition of unmet need by payers
 - ~ 55% Medicaid, 40% commercial, 5% Medicare/other¹
- Pricing precedent for exon skippers

Capital-Efficient Commercial Infrastructure Designed to Deliver Potentially Transformative Therapies and Optimize Value



Experienced launch team assembled for targeted execution

- Strong expertise in rare neurological and neuromuscular diseases
 - Broad US launch experience including SPINRAZA, ELEVIDYS, VYONDYS, AMONDYS, and DAYBUE
- Deep expertise across Medical, Commercial, Value & Access, Manufacturing, Program Management, Patient Advocacy and Services, Government Affairs, Digital, Data & Analytics



Ongoing education & awareness building

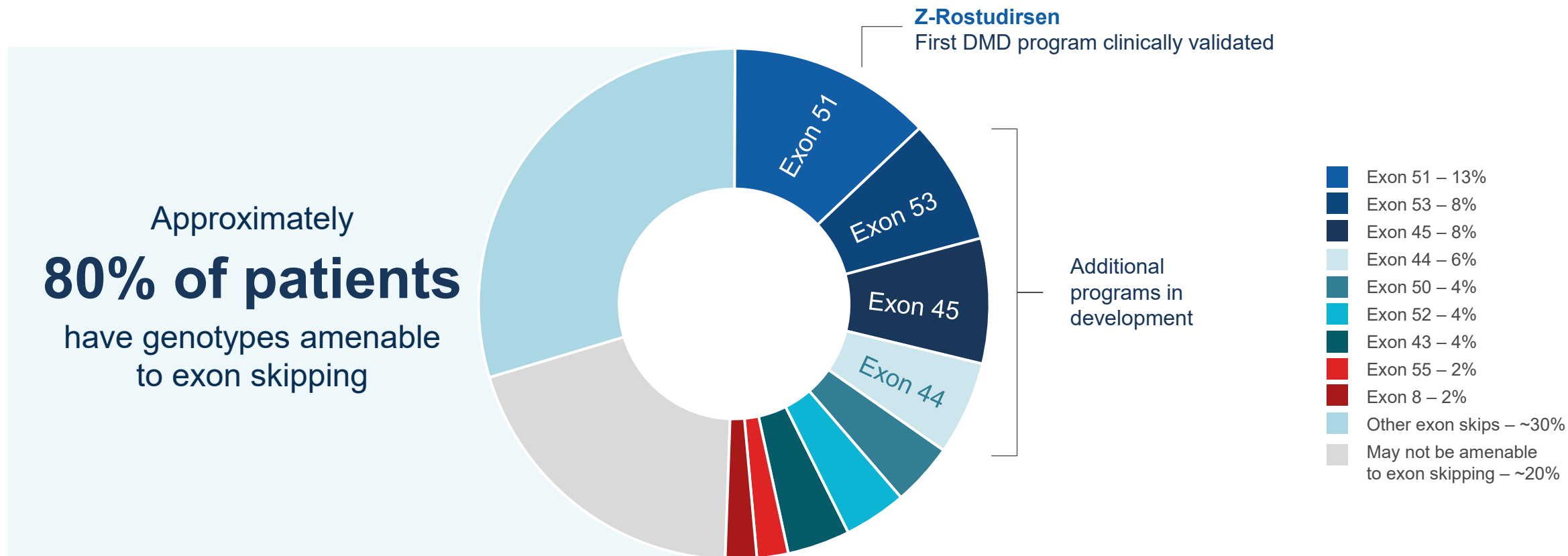
- Site of care visits underway across Medical and Commercial field teams
- Ongoing engagement with payer community
- > 5 years of sustained patient community and advocacy organization engagement



Manufacturing capabilities and team ready to supply DMD and DM1 potential launches

- Proven track record of providing drug supply across global clinical trials since 2022
- Leveraging same Fab and linker across DMD and DM1 programs
- Commercial-scale manufacturing partnerships in place with sufficient capacity and redundant, geographically-diverse suppliers

De-Risked Opportunity to Build a Broader DMD Franchise Potentially Tripling Addressable Patient Population



DMD franchise assets designed to leverage same Fab, linker, and payload chemistry as z-rostudirsen

Drivers for Potential Shareholder Value Creation in DMD

ATTRACTIVE MARKET CHARACTERISTICS

Significant
unmet needs despite
approved therapies



Identified
patient population



Pricing precedent
with established
reimbursement



Concentrated
treatment centers



POSITIONED TO MAXIMIZE VALUE

Wholly-owned
assets



Potential **best-in-class profile** for lead program



Capital-efficient
commercial model



De-Risked expansion opportunities



ON TRACK FOR Q1 2027 POTENTIAL LAUNCH

Team and capabilities to execute





Second Lead Program Leveraging Clinically Validated Platform for Larger Adjacent Indication: Z-Basivarsen in DM1



DM1 is a Devastating Neuromuscular Splicing Disorder



Population

- ~40,000 (US)
- ~55,000 (EU)



Overview

- Mutation in the *DMPK* gene leads to mis-splicing of multiple genes
- Onset at any point, depending on DM1 phenotype
- Life expectancy of 45 - 60 years



Clinical Presentation

- Muscle weakness & myotonia
- CNS manifestations including fatigue, cognition, and sleep
- Gastrointestinal issues
- Cardiac arrhythmia
- Pulmonary abnormalities



**NO
approved
therapies**

OUR APPROACH

Functional Improvement via Splicing Correction in Nucleus

Restore normal RNA splicing to achieve **functional improvement** for those living with DM1

ACHIEVE Study to Support Accelerated Approval of Z-Basivarsen in DM1



Selection of registrational dose (6.8 mg/kg Q8W) based on multiple ascending dose (MAD) data



Data support vHOT improvement as early indicator of clinical benefit with z-basivarsen



Proof-of-concept that z-basivarsen can reverse disease progression across multiple functional endpoints



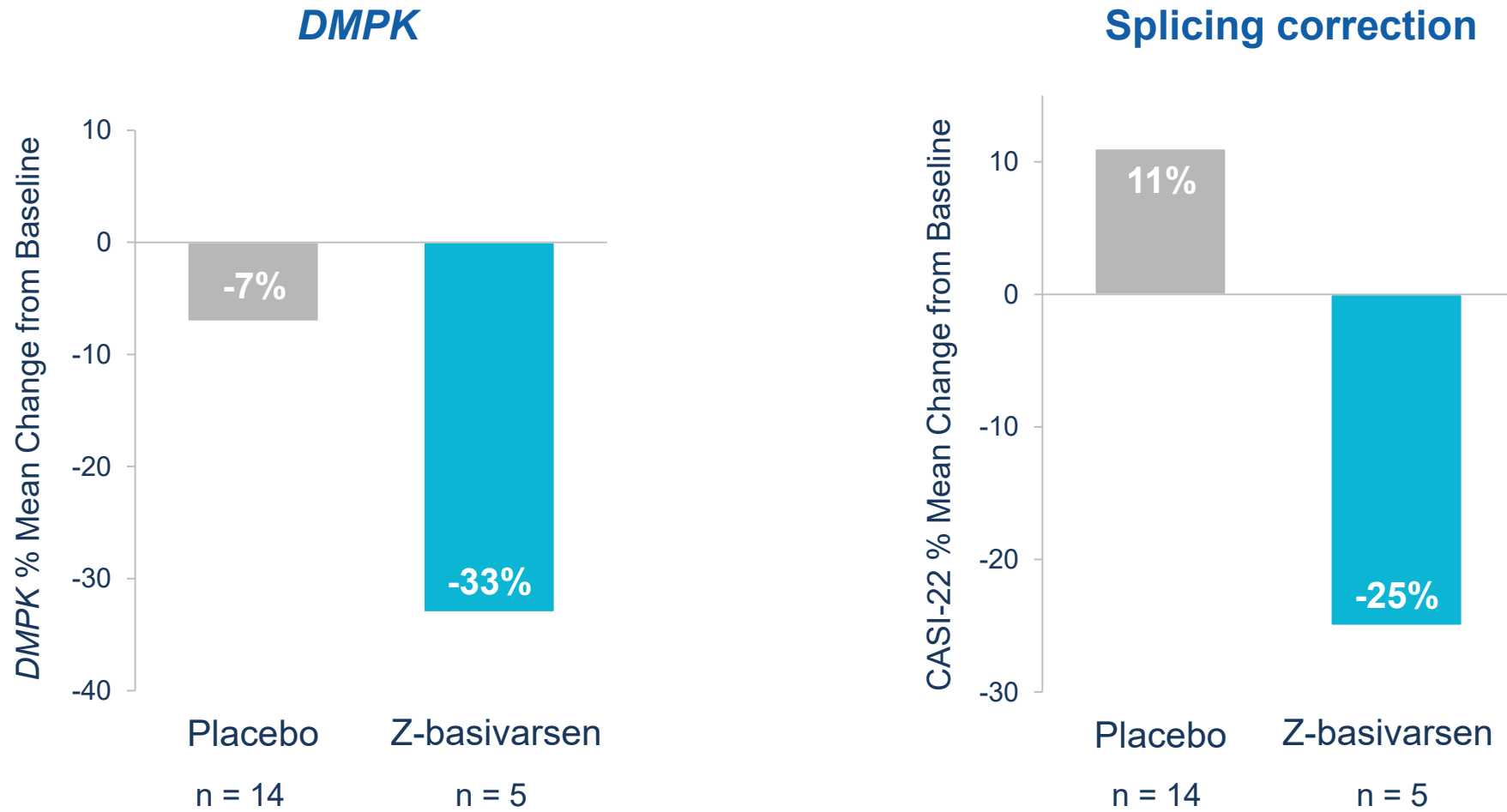
Favorable safety profile¹



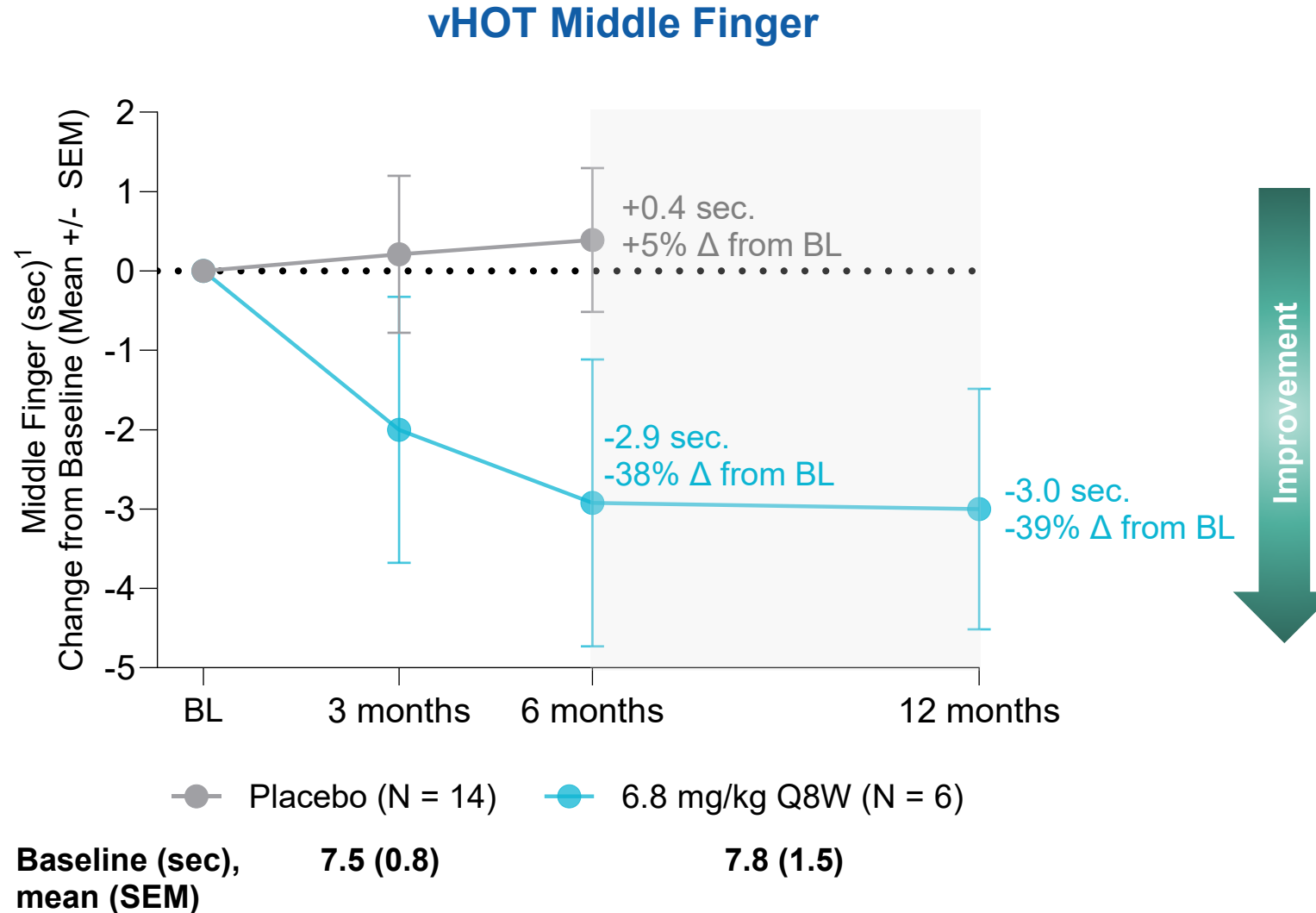
Potential submission for U.S. Accelerated Approval based on data from Registrational Expansion Cohort (REC)

Expect to complete enrollment of ACHIEVE REC in early Q2 2026
Phase 3 study planned to support full approval of z-basivarsen globally

Z-Basivarsen at 6.8 mg/kg Q8W Improved Foundational Pathobiology of DM1 at 3 Months

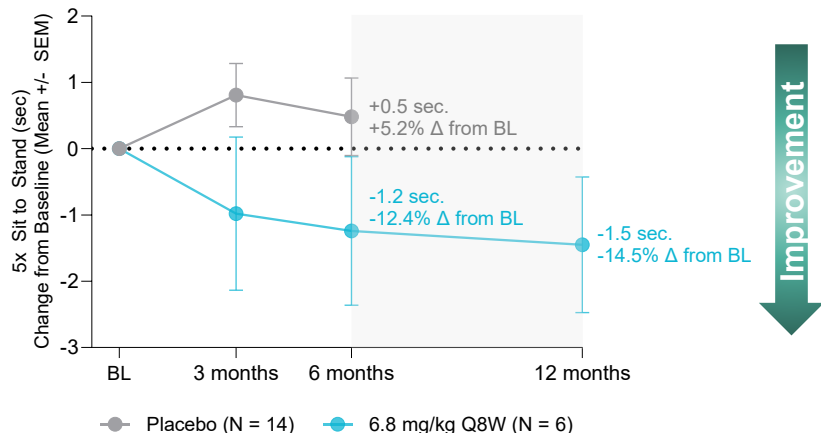


Robust and Sustained vHOT Improvement with Z-Basivarsen at 6 and 12 Months



Broad and Sustained Functional and Patient-Reported Improvement with Z-Basivarsen through 12 Months

5 Times Sit to Stand

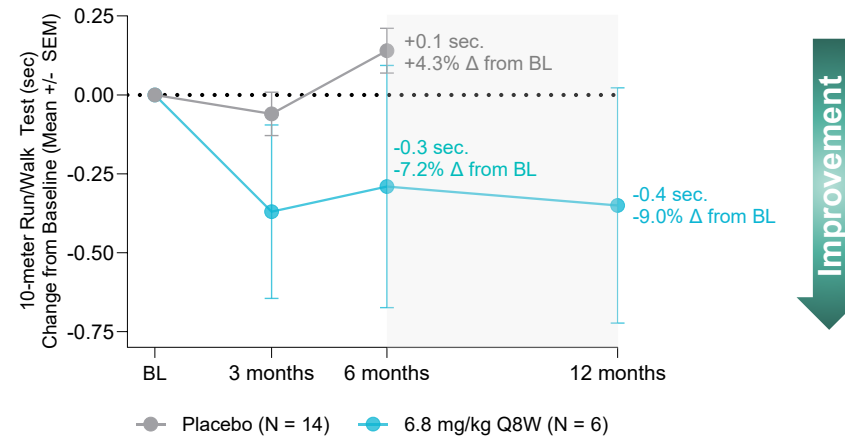


Baseline (sec), mean (SEM) 9.2 (0.5) 10.0 (1.4)

● Placebo (N = 14) ● 6.8 mg/kg Q8W (N = 6)



10-Meter Walk/Run Test

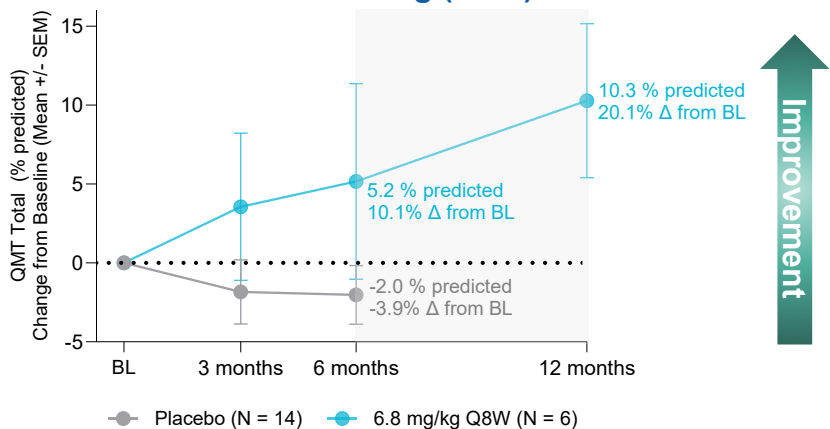


Baseline (sec), mean (SEM) 3.3 (0.1) 3.9 (0.6)

● Placebo (N = 14) ● 6.8 mg/kg Q8W (N = 6)



Quantitative Muscle Testing (QMT) Total Score

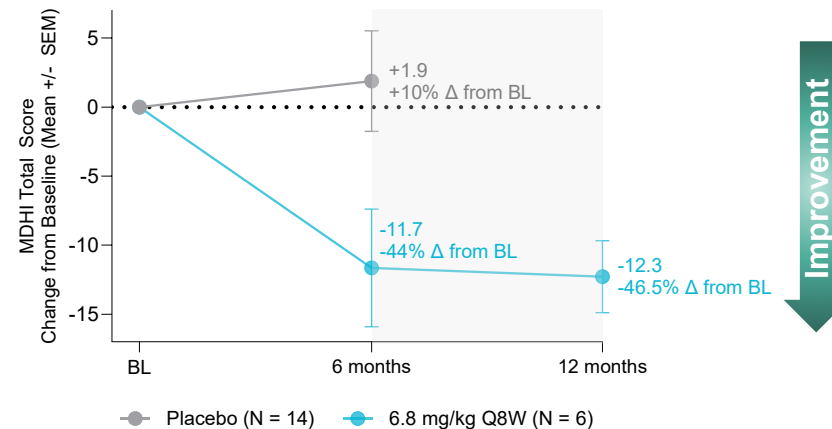


Baseline score, mean (SEM) 51.5 (3.8) 51.3 (4.2)

● Placebo (N = 14) ● 6.8 mg/kg Q8W (N = 6)



Myotonic Dystrophy Health Index (MDHI) Total Score

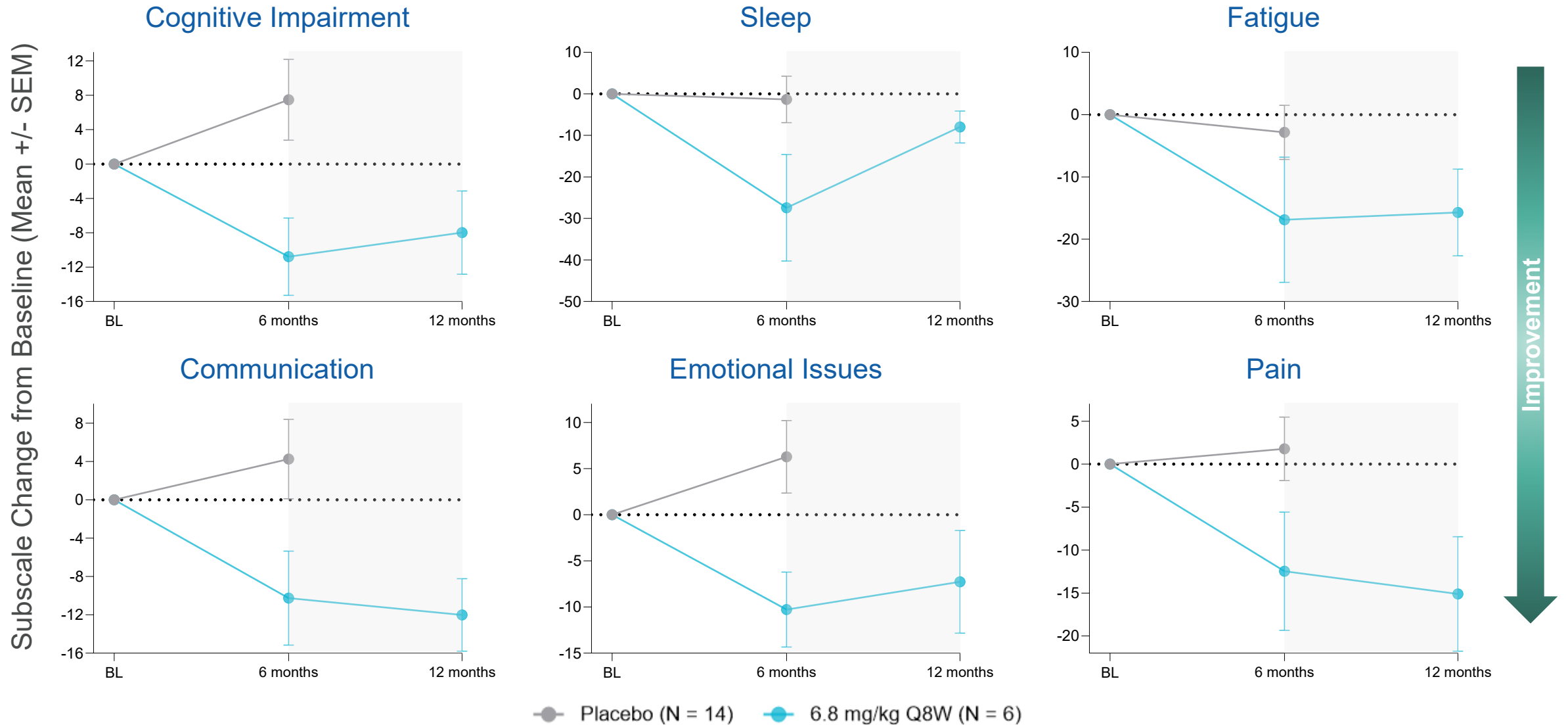


Baseline, mean (SEM) 18.7 (3.8) 26.5 (5.6)

● Placebo (N = 14) ● 6.8 mg/kg Q8W (N = 6)



Sustained Improvement in CNS-related MDHI Subscales



Z-Basivarsen: Favorable Safety Profile with No Serious Related TEAEs

Summary of Treatment Emergent Adverse Events (TEAEs)¹

TEAE Category	Participants with ≥1 TEAE – n (%)					
	1.8 mg/kg Q4W+Rec. N=16	3.4 mg/kg Q4W+Rec. N=16	3.4 mg/kg Q8W N=8	5.4 mg/kg Q8W N=8	6.8 mg/kg Q8W N=8	Overall (N=56)
Any TEAE	16 (100%)	16 (100%)	8 (100%)	8 (100%)	8 (100%)	56 (100%)
Any related TEAE	9 (56%)	10 (63%)	3 (38%)	6 (75%)	6 (75%)	34 (61%)
Any serious TEAE	4 (25%)	0	1 (13%)	0	0	5 (9%)
Any serious related TEAE	0	0	0	0	0	0
Any TEAE leading to withdrawal from study	0	0	0	0	0	0
Any TEAE leading to death	0	0	0	0	0	0

Most TEAEs Were Mild or Moderate in Intensity¹




- 6 serious TEAEs unrelated to study drug
 - Atrioventricular block first degree (1)²
 - Pneumonia (2 events in same participant)
 - Pulmonary embolism (1)³
 - Hyponatremia (1)
 - Influenza (1)
- Most common TEAEs (≥20% participant incidence)⁴
 - Nasopharyngitis (41%)
 - Procedural pain (34%)
 - Influenza (30%)
 - Infusion-related reaction (29%)
 - Headache (27%)
 - Diarrhea (23%)

Additional Safety Data

- Liver enzyme elevations have been observed in a minority of participants
 - No impact on liver function (bilirubin or coagulation)
 - Interpretation is complicated by underlying disease and elevated baseline values up to ~2.5x greater than the upper limit of normal
- No participants have demonstrated persistent related anemia or thrombocytopenia

~1000 Doses of Study Drug Administered to Date Representing 93 Patient-Years of Follow-Up¹

Transforming Dyne into a Commercial Organization as Early as 2027

Z-Rostudirsen for Exon 51 DMD			Z-Basivarsen for DM1	
Q1 2025	Completed enrollment of Registrational Expansion Cohort		Early Q2 2026	Complete enrollment planned for Registrational Expansion Cohort
December 2025	Positive topline results from Registrational Expansion Cohort		Q1 2027	Data planned for Registrational Expansion Cohort
Q2 2026	Planned submission for U.S. Accelerated Approval		Early Q3 2027	Potential submission for U.S. Accelerated Approval
Q1 2027	Potential U.S. launch, assuming Priority Review		Q1 2028	Potential U.S. launch, assuming Priority Review

One capital efficient operating model to support multiple potential commercial launches



Q&A

